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Experiences of Structured Elicitation for Model-Based Cost-Effectiveness Analyses

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ABSTRACT

Background: Empirical evidence supporting the cost-effectiveness estimates of particular health care technologies may be limited, or it may even be missing entirely. In these situations, additional information, often in the form of expert judgments, is needed to reach a decision. There are formal methods to quantify experts' beliefs, termed as structured expert elicitation (SEE), but only limited research is available in support of methodological choices. Perhaps as a consequence, the use of SEE in the context of cost-effectiveness modelling is limited. **Objectives:** This article reviews applications of SEE in cost-effectiveness modelling with the aim of summarizing the basis for methodological choices made in each application and recording the difficulties and challenges reported by the authors in the design, conduct, and analyses. **Methods:** The methods used in each application were extracted along with the criteria used to support methodological and practical choices and any issues or challenges discussed in the text. Issues and challenges were extracted using an open field, and then categorised and grouped for reporting. **Results:** The review demonstrates considerable heterogeneity in methods used, and authors acknowledge great methodological uncertainty in justifying their choices. Specificities of the context area emerging as potentially important in determining further methodological research in elicitation are between-expert variation and its interpretation, the fact that substantive experts in the area may not be trained in quantitative subjects, that judgments are often needed on various parameter types, the need for some form of assessment of validity, and the need for more integration with behavioural research to devise relevant debiasing strategies. **Conclusions:** This review of experiences of SEE highlights a number of specificities/constraints that can shape the development of guidance and target future research efforts in this area.

Keywords: Bayesian, cost effectiveness, decision modeling, elicitation, expert judgment, subjective.

Introduction

Reimbursement decisions are often supported by model-based economic evaluation (MBEE) [1]. Uncertainty in the evidence used to populate these models can result in uncertain cost-effectiveness estimates [2]. There may be circumstances in which empirical data are limited (e.g., a cancer product licensed on the basis of progression-free survival, with limited evidence on survival impacts) or are missing entirely (e.g., when assessing the value of a future clinical trial for a medical technology). In these situations, additional information, often in the form of expert judgments, reported as a distribution, is needed to reach a decision. To improve the accountability of the decision-making process, the procedure used to derive these judgments should be transparent, with any uncertainty in individual judgments characterized, in addition to between-expert variation [3].

Formal methods to quantify experts' beliefs exist and are termed as structured expert elicitation (SEE) [3,4]. Elicitation has been used in various disciplines including weather forecasting [5] and food and safety risk assessments [6]. Nevertheless, the existing methodological research on elicitation, both generic and discipline-specific, is inconsistent and noncommittal [7]. Methodological uncertainties may be one of the main reasons for the limited use of formal SEE in the context of MBEE. A review of applications in this area, published in 2013 [8], identified only a small number (14) of studies reporting the use of SEE. This review did not seek to determine the reasons for heterogeneity of approach, nor did it look at the challenges faced when conducting SEE to support MBEE and inform directions for future research.

In pursuit of further clarity, this article updates the aforementioned review [8], but instead of reporting the way elicitation is being used in practice, it focuses on summarizing the basis for

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Table 1 – Summary of applications.

Study	Type of strategy under investigation	Was the aim to inform an early assessment (i.e., R&D) rather than reimbursement?	Type of parameter(s) elicited
Garthwaite et al. [14]	Treatment	No	Event probabilities, time to event, dependency
Leal et al. [10]	Diagnostic/screening	No	Event probabilities, relative effectiveness, diagnostic accuracy
Girling et al. [15]	Treatment	Yes	Event probabilities, time to event
Stevenson et al. [16]	Prevents transmission	No	Event probabilities, time to event, relative effectiveness
Meads et al. [12]	Diagnostic/screening	Yes	Event probabilities, diagnostic accuracy, minimum important clinical difference
McKenna et al. [19]	Treatment	No	Event probabilities
Haakma et al. [13]	Diagnostic/screening	Yes	Diagnostic accuracy
Stevenson et al. [17]	Treatment	No	Event probabilities, relative effectiveness
Speight et al. [25]	Diagnostic/screening	No	Event probabilities
Sperber et al. [22]	Treatment	No	Event probabilities, relative effectiveness
Brodtkorb [26]	Several exercises conducted but insufficient detail reported on each		
Colbourn et al. [28]	Diagnostic/screening	No	Event probabilities, relative effectiveness
Soares et al. [9]	Treatment	No	Event probabilities, relative effectiveness
Bojke et al. [18]	Treatment	No	Relative effectiveness, dependency
Cao et al. [11]	Diagnostic/screening	Yes	Relative effectiveness
Fischer et al. [23]	Treatment	No	Counts, time to event
Poncet et al. [27]	Diagnostic/screening	No	Event probabilities
Grigore et al. [24]	Treatment	No	Event probabilities
Wilson et al. [20]	Treatment	No	Event probabilities, relative effectiveness
Meeyai et al. [21]	Vaccine	No	Event probabilities
Grimm et al. [35]	Diagnostic/screening	No	Diffusion[†]

R&D, research and development.
[†] Rate of implementation in clinical practice over time.

methodological choices made in each application (design, conduct, and analysis) and the difficulties and challenges reported by the authors. In the Methods section, the methods for identifying the literature are described and an overview of the contexts in which SEE was used across studies is made. The sections that follow discuss choices, challenges, and issues relating to the design of SEE; conduct of SEE; and analyses of SEE. In detailing these elements it is necessary to first describe the applications (see the Summary of Applied Studies section and Tables 1–3), and that is where the similarities exist between this review and the 2013 [8] review, and also where they end. The last section sets out specific challenges posed by SEE in MBEE to inform the direction of future research.

Methods

To identify applications of SEE, the 2013 review [8] was updated (identifying studies up to April 11, 2017). Further details on the methods of the search are given in the Appendix in Supplemental Materials found at https://doi.org/10.1016/j.jval.2018.01.019, but, in brief, studies were identified via Ovid SP MEDLINE and, similarly in the 2013 review [8], were included only if they contained an SEE to elicit uncertain parameters (in the form of a distribution) to inform MBEE. Studies conducting preference elicitation(e.g., to generate utility estimates for health states) were not included.

The methods used in each application were extracted (the extraction form is reproduced in Tables 1–3, which also present results) along with the criteria used to support methodological and practical choices and any issues or challenges discussed in the text. Issues and challenges were extracted using an open field and then categorized and grouped for reporting.

Results

Summary of Applied Studies

In total, 21 studies were included. Table 1 and the Appendix in Supplemental Materials provide summary information on each study and highlight that elicitation has been used mainly when data on a particular parameter are limited or absent. Four of the 21 applications were applied in an early modeling context, where there may not be direct clinical experience with the technology of interest, and 8 evaluated a diagnostic or screening strategy.

Table 2 presents the method of recruiting experts, methods of elicitation, and methods of aggregation in each of the applied studies. Table 3 presents how the SEE was conducted, including mode of administration and use of any software, and also any analyses that were performed. Each element of the applied studies is considered, and choices, challenges, and issues discussed in the following sections.

Aspects Related to the Design of the SEE

Considerations on the design of the SEE were grouped according to specification of the quantities to elicit, selection of experts, elicitation method, and type of aggregation and weighting of experts' judgments.

Specification of quantities to elicit

In all applications, experts' beliefs were sought for only a few parameters of a decision model, often not elicited directly but calculated from one or more alternative elicited quantities. For example, a time-constant transition probability could be